Evaluating the **Safety** & **Efficacy** of Drugs for Rare Diseases or Conditions in the United States and the European Union

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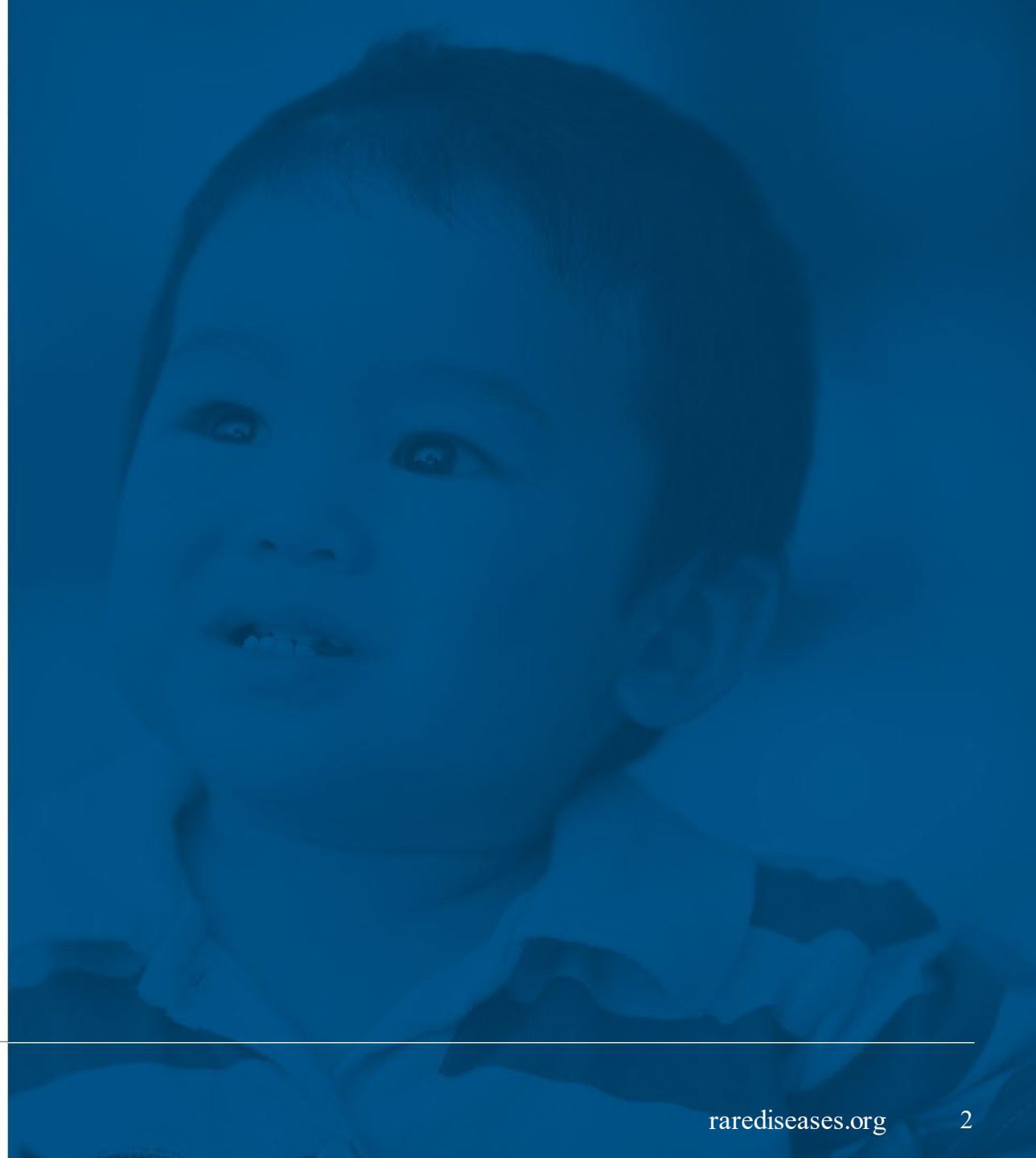
NORD® MISSION STATEMENT

We improve the health and well-being of people with rare diseases by driving advances in care, research, and policy.



CURRENT CHALLENGES IN RARE DISEASE DRUG DEVELOPMENT

NO drugs for >95% of all rare diseases

Approved drugs often struggle to **stay** on market

Patients struggle to **access** approved therapies

NORD's hope for this NASEM study:

1. Strengthen global harmonization
2. Protect what currently works
3. Identify best practices & lessons learned

PATIENTS & FAMILIES PLAY A KEY ROLE IN RARE DISEASE DRUG DEVELOPMENT

It's estimated that

25-30

MILLION AMERICANS

(almost 1 in 10) have rare diseases.[7]

More than

90%

of rare diseases are **WITHOUT** an FDA-approved treatment.[9]

Many rare diseases result in premature deaths of infants and young children, or are fatal in early childhood.[4]

Most rare diseases are **genetic** or have a genetic component.[3]

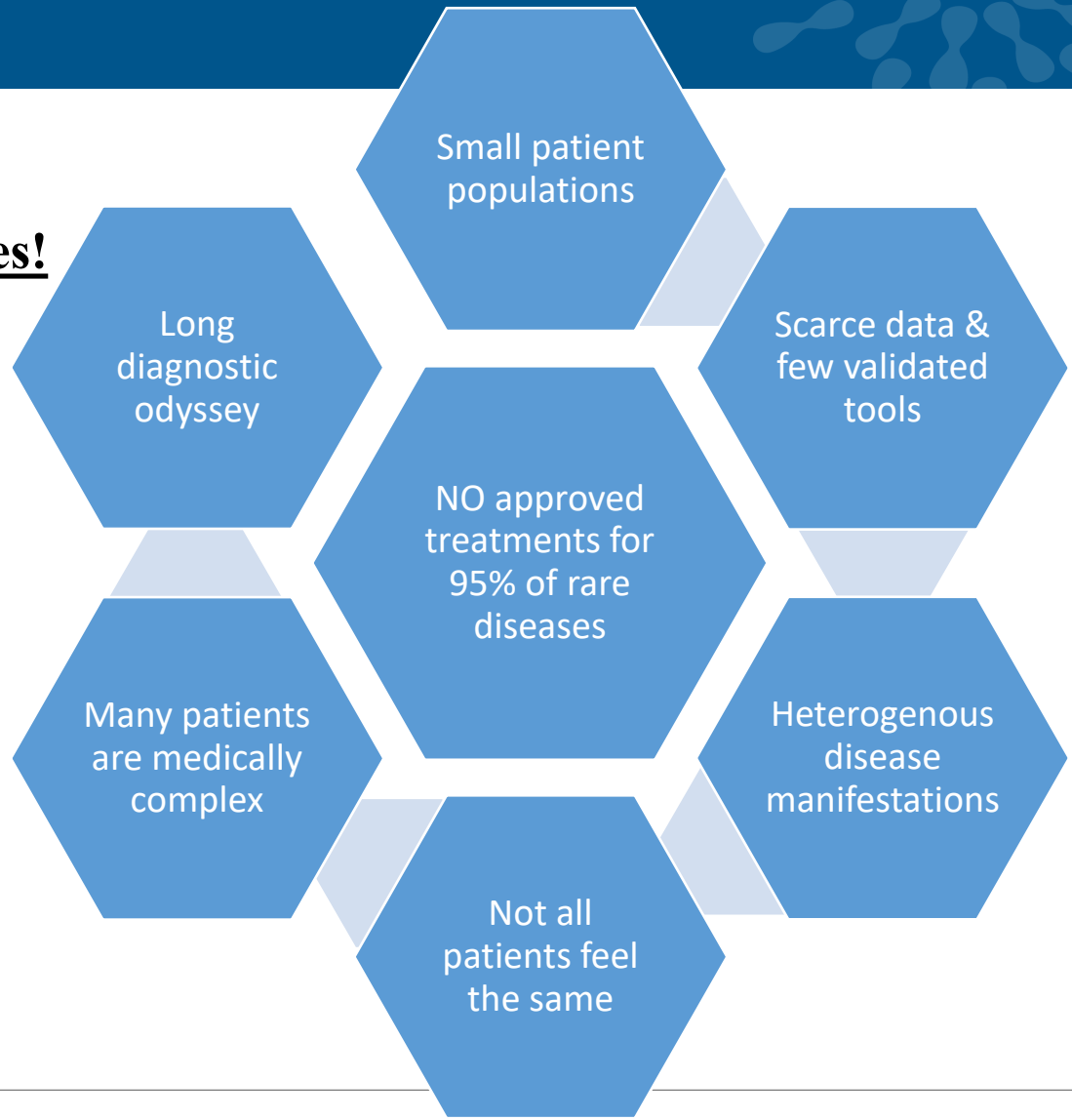
- Most rare disease patients have **NO FDA-APPROVED TREATMENTS** and **NO CURES**
- Many rare diseases have a **GENETIC** component and lead to **PREMATURE DEATH** in **CHILDHOOD**
- Most rare diseases have heterogenous **DISEASE MANIFESTATIONS** and **PROGRESSIONS**
- Cell & gene therapies are the only hope for a cure for many **RARE DISEASE PATIENTS**
- Effective treatments have completely changed the **DISEASE TRAJECTORY** for patients and families

KEY CHALLENGES IN RARE DISEASE DRUG DEVELOPMENT

<u>Rare disease patients need more safe & effective therapies!</u>

Rare Disease Drug Development:

1. The path to market takes too much time & money
2. Largely done by start-ups & pre-revenue companies
3. Often not in synch with coverage & reimbursement



RARE DISEASES, LIMITED PATIENT POPULATIONS, & GLOBAL CLINICAL TRIALS

Increasing **international harmonization** is key for rare disease drug development

Technical requirements (ICH)

Data & registries

Patient advocacy, mutual recognition agreements, etc. ….

ORPHAN DRUG DEVELOPMENT SIMILARITIES & DIFFERENCES IN THE U.S. / E.U.

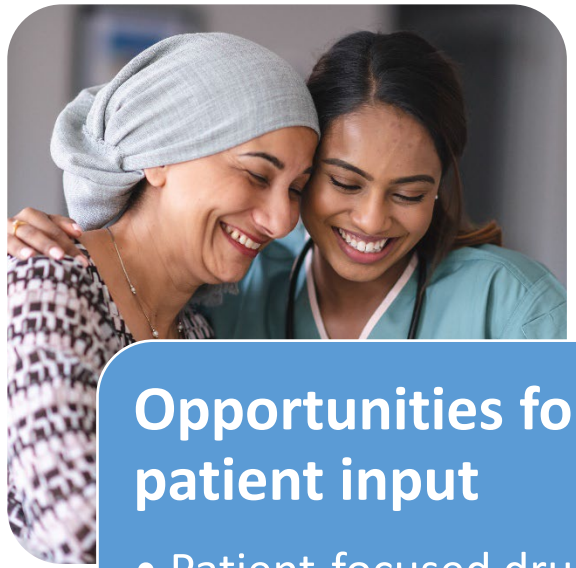
What is a rare disease

- Prevalence threshold
- Data requirements
- Market exclusivity

- **Predictability is key!**

Initiatives to support sponsors

- Early interactions
- Expedited programs
- Other support programs (e.g., RDEA, ARC)



Opportunities for patient input

- Patient-focused drug development (PFDD)
- Patient experience data (e.g., PROMs)
- Clinical trial design, …

OTHER ISSUES IMPACTING ORPHAN DRUG DEVELOPMENT

Data & standards

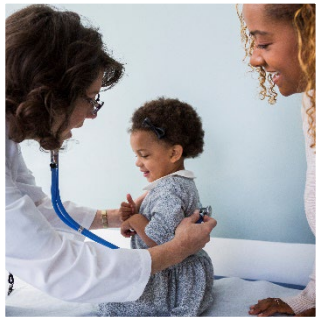
Special populations

Capturing value

A FEW SPECIFIC EXAMPLES TO STRENGTHEN GLOBAL HARMONIZATION

International patient registries

Global data sharing platforms



A global view on PROMs, ClinROs, etc.

Improve alignment & international collaboration to overcome key rare disease challenges, e.g.

- **Unknown natural history & disease progression**
- **Limited patient populations & scarce data**
- **Clinical trials that do not measure key outcomes that matter to patients**
- **Etc. etc.**

THANK YOU

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